



4160-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2013-D-0811]

Guidance for Industry: Enforcement Policy Regarding Investigational New Drug Requirements for Use of Fecal Microbiota for Transplantation to Treat Clostridium difficile Infection Not Responsive to Standard Therapies; Availability

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA or Agency) is announcing the availability of a guidance for industry entitled “Enforcement Policy Regarding IND Requirements for Use of Fecal Microbiota for Transplantation to Treat Clostridium difficile Infection Not Responsive to Standard Therapies,” dated July 2013. This guidance informs members of the medical and scientific community and other interested persons that we intend to exercise enforcement discretion regarding the investigational new drug (IND) requirements for the use of fecal microbiota for transplantation (FMT) to treat C. difficile infection not responding to standard therapies. FDA intends to exercise this discretion provided that the treating physician obtains adequate informed consent from the patient or his or her legally authorized representative for the use of FMT products. Informed consent should include, at a minimum, a statement that the use of FMT products to treat C. difficile is investigational and a discussion of its potential risks. This policy does not extend to other uses of FMT. FDA intends to exercise this discretion on an interim basis while we further consider the matter. This guidance has an

immediate implementation date because FDA has determined that prior public participation is not feasible or appropriate.

DATES: Submit either electronic or written comments on Agency guidances at any time.

ADDRESSES: Submit written requests for single copies of the guidance to the Office of Communication, Outreach and Development (HFM-40), Center for Biologics Evaluation and Research (CBER), Food and Drug Administration, 1401 Rockville Pike, suite 200N, Rockville, MD 20852-1448. Send one self-addressed adhesive label to assist that office in processing your requests. See the SUPPLEMENTARY INFORMATION section for electronic access to the guidance document.

Submit electronic comments on the guidance to <http://www.regulations.gov>. Submit written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852.

FOR FURTHER INFORMATION CONTACT: Paul E. Levine, Jr., Center for Biologics Evaluation and Research (HFM-17), Food and Drug Administration, 1401 Rockville Pike, suite 200N, Rockville, MD 20852-1448, 301-827-6210.

SUPPLEMENTARY INFORMATION:

I. Background

FDA is announcing the availability of a guidance for industry entitled “Enforcement Policy Regarding IND Requirements for Use of Fecal Microbiota for Transplantation to Treat Clostridium difficile Infection Not Responsive to Standard Therapies,” dated July 2013. This guidance is being issued consistent with FDA’s good guidance practices (GGP) regulation § 10.115 (21 CFR 10.115). This guidance is being implemented without prior public comment because the Agency has determined that prior public participation is not feasible or appropriate

(§ 10.115(g)(2)). The Agency made this determination because the guidance requires immediate implementation for public health reasons. This guidance deals with an urgent issue affecting patients with life-threatening infections with C. difficile. Although this guidance document is immediately in effect, it remains subject to comment in accordance with the Agency's GGP's regulation.

Fecal microbiota collected from healthy individuals are being investigated for use in the treatment of C. difficile infection. Published data suggest that the use of fecal microbiota to restore intestinal flora may be an effective therapy in the management of refractory C. difficile infection. However, the efficacy and safety profile of this intervention have not yet been fully evaluated in controlled clinical trials.

In the Federal Register of February 25, 2013 (78 FR 12763), FDA announced a public workshop, entitled "Fecal Microbiota for Transplantation," which was held on May 2 and 3, 2013. The purpose of the workshop was to provide a forum for the exchange of information, knowledge, and experience among the medical and scientific community about the regulatory and scientific issues associated with FMT. During that workshop, and in subsequent communications, physicians and scientists expressed concern to FDA that FMT is not appropriate for study under the Agency's IND regulations (21 CFR part 312). Some health care providers stated that applying IND requirements will make FMT unavailable and suggested that an alternative regulatory approach is needed to ensure the widespread availability of FMT for individuals with C. difficile infection unresponsive to standard therapies.

FDA acknowledges these concerns. The Agency intends to exercise enforcement discretion regarding the IND requirements for the use of FMT to treat C. difficile infection not responding to standard therapies, provided that the treating physician obtains adequate informed

consent from the patient or his or her legally authorized representative for the use of FMT products. Informed consent should include, at a minimum, a statement that the use of FMT products to treat C. difficile is investigational and a discussion of its potential risks. FDA intends to exercise this discretion on an interim basis while the Agency further considers the matter.

This policy does not extend to other uses of FMT. Data related to the use and study of FMT to treat diseases or conditions other than C. difficile infection are limited, and study of FMT for these other uses is not included in this enforcement policy.

This guidance represents the Agency's current thinking on this topic. It does not create or confer any rights for or on any person and does not operate to bind FDA or the public. An alternative approach may be used if such approach satisfies the requirements of the applicable statutes and regulations.

II. Comments

Interested persons may submit either electronic comments regarding this document to <http://www.regulations.gov> or written comments to the Division of Dockets Management (see ADDRESSES). It is only necessary to send one set of comments. Identify comments with the docket number found in brackets in the heading of this document. Received comments may be seen in the Division of Dockets Management between 9 a.m. and 4 p.m., Monday through Friday, and will be posted to the docket at <http://www.regulations.gov>.

III. Electronic Access

Persons with access to the Internet may obtain the guidance at either <http://www.fda.gov/RegulatoryInformation/Guidances/default.htm> or <http://www.regulations.gov>.

Dated: July 12, 2013.

Leslie Kux,

Assistant Commissioner for Policy.

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